

Figure 1

Wild type 3' LTR sequence:

- I GAACAGATGG AACAGCTGAA TATGGGCCAA ACAGGATATC TGTGG TAAGC
- 51 AGTTCC TGCC CCGGCTCAGG GCCAAGAACA GATGGAACAG CTGAATATGG
- 101 GCCAAACAGG ATATCTGTGG TAAGCAGTTC CTGCCCCGGC TCAGGGCCAA
- 151 GAACAGATGG TCCCCAGATG CGGTCCAGCC CTCAGCAGTT TCTAGAGAAC
- 201 CATCAGATGT TTCCAGGGTG CCCCAAGGAC CTGAAATGAC CCTGTGCCTT
- 251 ATTTGAACTA ACCAATCAGT TCGCTTCTCG CTTCTGTTCG CGCGCTTCTG
- 301 CTCCCCGAGC TCAATAAAA

Corresponding modified 3' LTR sequence from pENZ1 (modified sequences in bold italics):

- 1 ACGCTTGATC CGGCTACCTG CCCATTCGAC CACCAAGCGA AACATCGCAT
- 51 CGAGCGAGCA CGTACTCGGA TGGAAGCCGG TCTTGTCGAT CAGGATGATC
- 101 TGGACGAAGA GCATCAGGGG CTCGCGCCAG CCGAACTGTT CGCCAGGCTC
- 151 AAGGCGCGCA TGCCCGACGG CGAGGATCTC GTCGTGACTT TCTAGAGAAC
- 201 CATCAGATGT TTCCAGGGTG CCCCAAGGAC CTGAAATGAC CCTGTGCCTT
- 251 ATTTGAACTA ACCGGTCAGT TCGCTTCTCG CTTCTGTTCG CGCGCTTCTG
- 301 CTCCCGAGC TCAGCTGCG

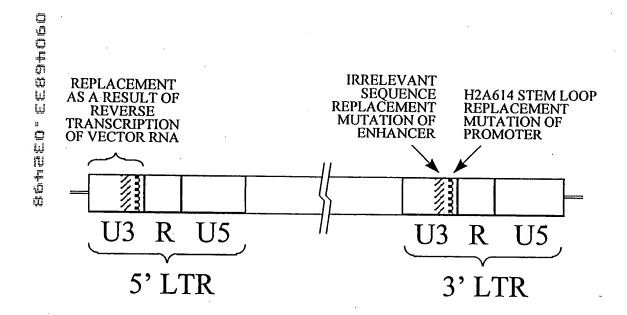


Figure 3

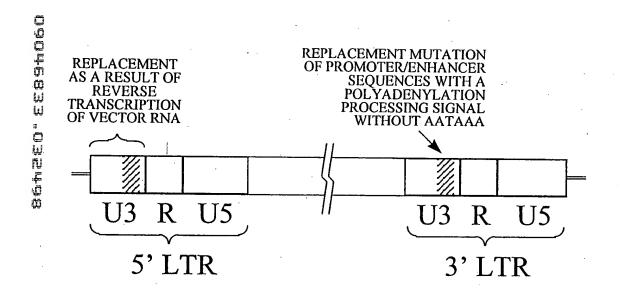


Figure 4

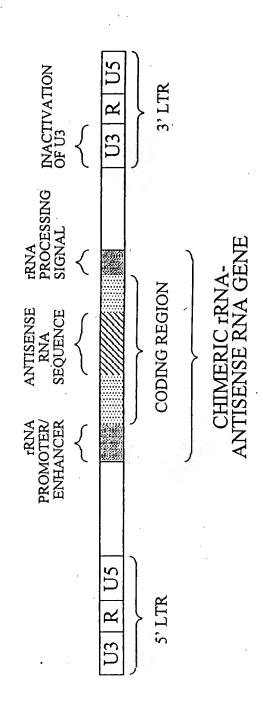


Figure 5

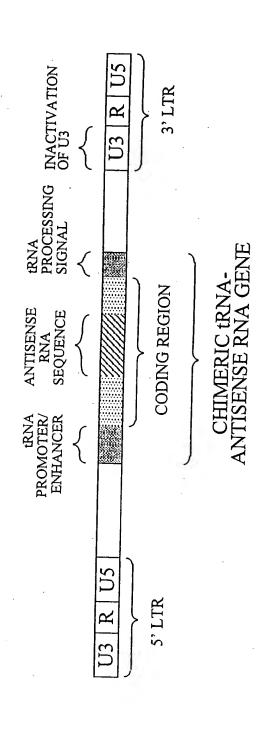


Figure 6

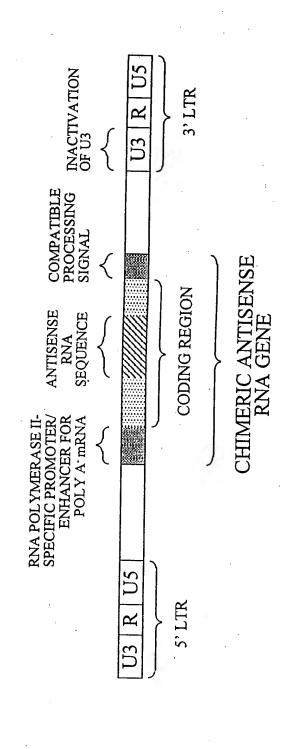
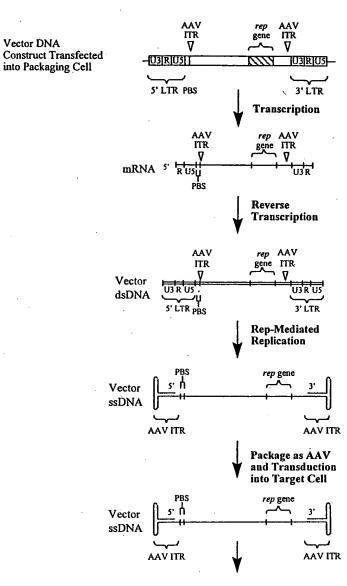


Figure 7

Vector DNA



Integration at q13.4-ter on Chromosome 19 of **Host Genome**

Figure 8

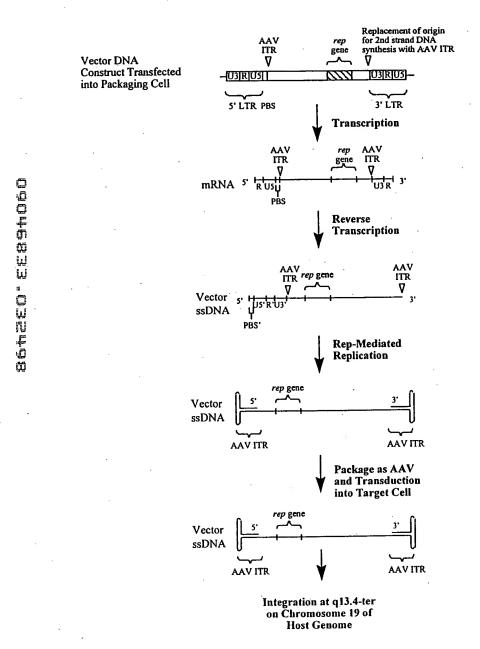


Figure 9

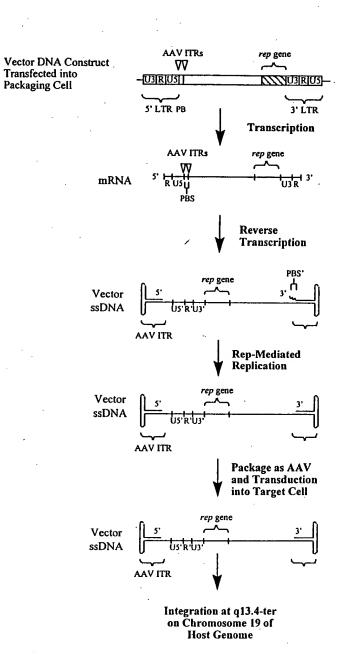


Figure 10

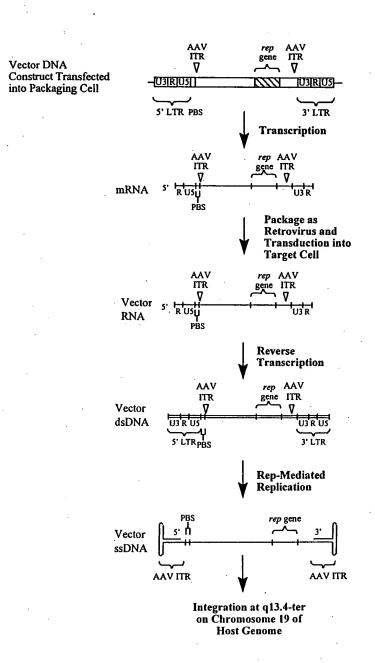
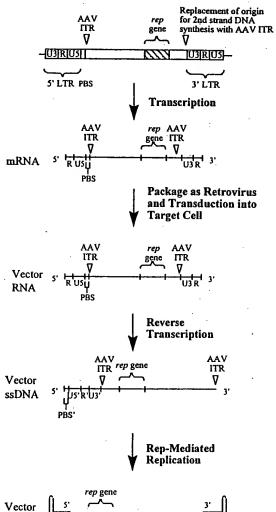
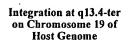


Figure 11





ssDNA

AAV ITR

Figure 12

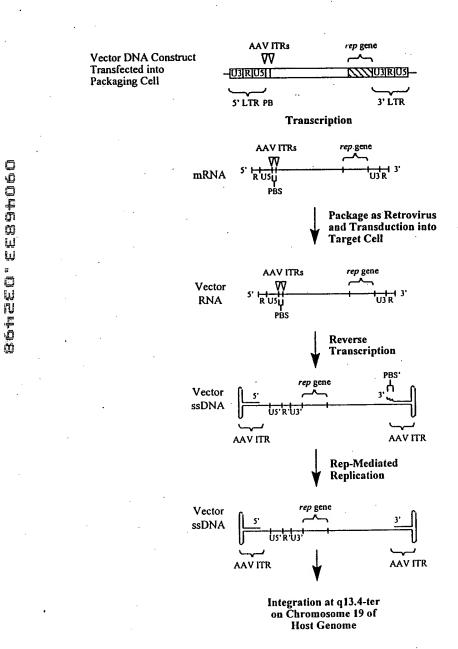


Figure 13